



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EMA/COMP/444415/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tariquidar for the treatment of P-gp positive breast cancer

On 21 March 2012, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for tariquidar for the treatment of P-gp positive breast cancer. A negative decision was issued by the European Commission on 27 July 2012.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition, as well as an assumption of potential benefit over currently available methods of treatment.

The negative opinion was based on the following reasons:

- The orphan condition applied for is the 'treatment of P-gp positive breast cancer'. With this condition the sponsor was specifically targeting breast cancer that is multidrug resistant due to high levels of P-gp. P-gp (or P-glycoprotein) is a protein used to transport a variety of substances in and out of cells. In breast cancer, P-gp is known to transport cytotoxic (cell killing) medicines out of cancer cells before they can have their effect, thereby making the cancer resistant to treatment. The proposed orphan condition refers to a subset of a broader medical condition, namely breast cancer, which is not rare. The sponsor claimed that the proposed condition covers only 25% of P-gp positive breast cancers as this is the proportion they expect to be multidrug resistant. However, the COMP noted that P-gp may be found in close to 100% of breast cancer patients, and that tariquidar could therefore have an effect in a much larger population group than the subset proposed by the sponsor.
- The COMP considered the 25% cut-off point to be arbitrary and unsupported by evidence, and concluded that the condition applied for (treatment of P-gp positive breast cancer) is not a valid subset for orphan designation. Therefore, the prevalence of the broader medical condition (breast cancer) should have been taken into account. This is more than 5 in 10,000 people in the European Union and therefore above the ceiling for orphan designation.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.



For more information:

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Tariquidar	Treatment of P-gp positive breast cancer
Bulgarian	Тариквидар	Лечение на P-GP позитивен карцином на гърдата
Czech	Tarichidar	Léčba P-gp pozitivního karcinomu prsu
Danish	Tariquidar	Behandling af P-gp-positiv brystkræft
Dutch	Tariquidar	Behandeling van P-gp-positieve borstkanker
Estonian	Tarikvidaar	P-gp-positiivse rinnanäärmevähi ravi
Finnish	Tarikidaari	P-gp-positiivisen rintasyövän hoito
French	Tariquidar	Traitement du cancer du sein Pgp positif
German	Tariquidar	Behandlung von P-gp-positivem Brustkrebs
Greek	Ταρικιδάρη	Θεραπεία του θετικού στην p-γλυκοπρωτεΐνη καρκίνου του μαστού
Hungarian	Tarikvidar	Kezelése P-GP pozitív emlőrákban
Italian	Tariquidar	Trattamento del carcinoma mammario esprime P-gp
Latvian	Tarikvidars	p-gp pozitīva krūts vēža ārstēšana
Lithuanian	Tarikvidaras	Krūties vėžio su p-gp raiška gydymas
Maltese	Tariquidar	Trattament ta' P-gp Kanċer tas-Sider pożittivi
Polish	Tariquidar	Leczenie P-gp pozytywnego raka sutka
Portuguese	Tariquidar	Tratamento do cancro da mama P-gp positivo
Romanian	Tariquidar	Tratamentul cancerului mamar P-gp pozitiv
Slovak	Tarichidar	Liečba P-gp pozitívneho karcinómu prsníka
Slovenian	Tarikvidar	Zdravljenje raka dojke, pozitivnega za P-glikoprotein
Spanish	Tariquidar	Tratamiento del cáncer de mama positivo para P-gp
Swedish	Tarikidar	Behandling av P-gp-positiv bröstcancer

¹ At the time of designation